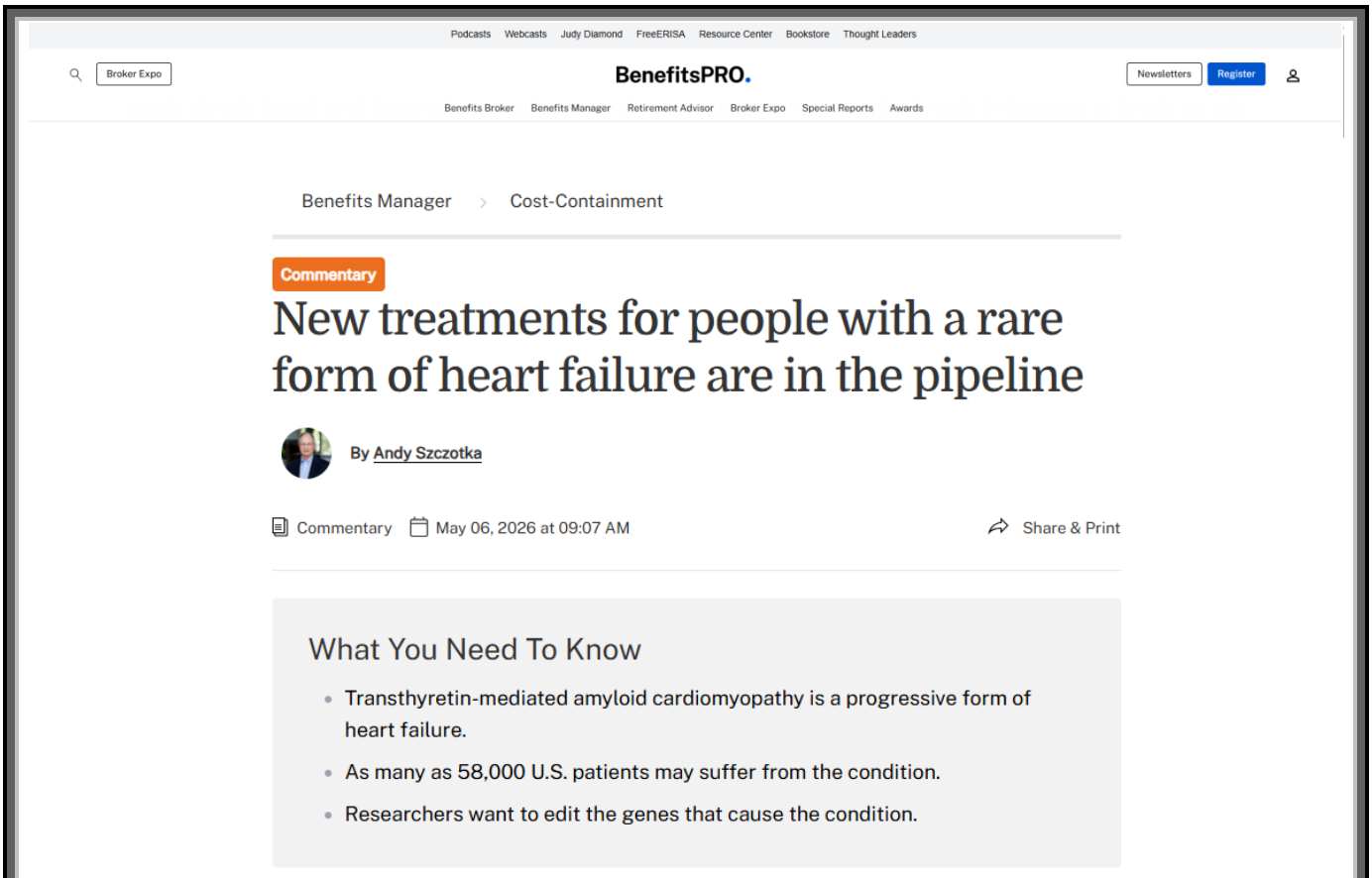

A new specialty drug coming to market can completely transform an employer's spend, with a single high-cost claim having the potential to upend a plan's entire budget.

Self-funded employers and their benefits advisors must become adept at identifying emerging specialty drug risk before it hits.

Employers and their advisors can do that by monitoring efforts to fight rare diseases like transthyretin-mediated amyloid cardiomyopathy, or ATTR-CM.

ATTR-CM

ATTR-CM is a progressive form of heart failure.

When patients have ATTR-CM, misfolded transthyretin proteins, or TTR proteins, accumulate in the heart, stiffening heart tissue and impairing the ability of the heart to relax and fill properly.

The condition may be hereditary or nonhereditary.

Current analyses suggest that roughly 5,800 to 58,000 people in the United States may be affected. That means that brokers may encounter cases in commercial plans.

ATTR-CM is difficult to identify and is often misdiagnosed as the more common kind of heart failure. The condition is often diagnosed at an advanced stage. It's typically treated with high-cost specialty drug therapies.

Before 2019, physicians usually treated the condition with standard heart failure medications, such as diuretics and beta blockers, which manage symptoms but don't slow disease progression.

That changed with the approval of disease-modifying therapies, including oral TTR stabilizers and, more recently, an injectable TTR "silencer" that reduces protein production at the genetic level. In clinical trials, the new therapies have reduced cardiovascular-related hospitalization rates and mortality.

The financial impact of ATTR-CM therapies is substantial. Annual wholesale acquisition costs for the new therapies range from about $250,000 to about $500,000 per patient.

Several considerations for brokers stand out.

Pharmacy versus medical benefit exposure is critical.

Oral stabilizers are generally covered under the pharmacy benefit, while the injectable silencer is administered by healthcare professionals and reimbursed under the medical benefit.

Misalignment between benefit channels can affect prior authorization, member cost-sharing, stop-loss arrangements, drug cost reimbursement and site-of-care management.

Understanding how the PBM and medical carrier coordinate coverage is essential to avoiding fragmented oversight.

Clinical differences aren't always clear-cut.

While the newer disease-modifying therapies target ATTR-CM through different mechanisms, the limited amount of comparative data available makes it difficult to determine whether the higher-cost agents are clinically superior.

In practice, brokers and employers must rely on utilization management, benefit design, prior authorization and step-therapy frameworks to help with coverage decisions.

Combination therapy questions persist.

The newer disease-modifying therapies have different modes of action (in other words: stabilizers and silencers) and act on different steps in disease biology. That leads some to ask about the idea of using both types of therapy at the same time.

However, current evidence supporting additive benefit is limited. Expert guidance advises against routine combination therapy.

Given the potential financial impact, brokers should monitor emerging data and counsel clients accordingly.

Pipeline therapies could shift trends further.

Companies have treatment strategies that involve gene-editing technology and the creation of anti-amyloid antibodies in late-stage development.

The new, high-tech treatments may supplement existing treatments, potentially increasing specialty and medical spend.

Benefits advisors who anticipate these developments can help clients implement proactive benefit strategies rather than react to surprise claims.

ATTR-CM may not affect large populations, but efforts to treat serious, rare diseases like ATTR-CM carry high financial sensitivity.

For the advisors, the question isn't whether a case will appear, but whether benefits strategies will be prepared when it does.

Understanding the clinical fundamentals, coverage dynamics and evidence gaps around ATTR-CM can help advisors guide clients proactively, before specialty exposure becomes a budget surprise.

That proactive approach can ensure that a client's patients receive appropriate treatments.

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